Life is Rare

JP Morgan Healthcare Conference 2026

JCR Pharmaceuticals Co., Ltd.

Shin Ashida
Chairman, President, and CEO

Hiroyuki Sonoda, Ph.D.
Director, Senior Managing Executive Officer

Shin Ashida (Founder)
Representative Director, Chairman, President and CEO

Hiroyuki Sonoda
Representative Director, incoming President
(as of Apr 1, 2026)

Disclaimer Regarding Forward-Looking Statement

- This material contains forecasts, projections, goals, plans, and other forward-looking statements regarding the Company's financial results and other data. Such forward-looking statements are based on the Company's assumptions, estimates, outlook, and other judgments made in light of information available at the time of disclosure of such statements and involve both known and unknown risks and uncertainties. Accordingly, forecasts, plans, goals, and other statements may not be realized as described, and actual financial results, success/failure or progress of development, and other projections may differ materially from those presented herein.

- Information concerning pharmaceuticals and medical devices (including those under development) contained herein is not intended as advertising or as medical advice.

50 Years of JCR Pharma: A Journey of Growth and Innovation


Head Office


Kobe API Plant


Bio Research Ctr.


Murotani Plant

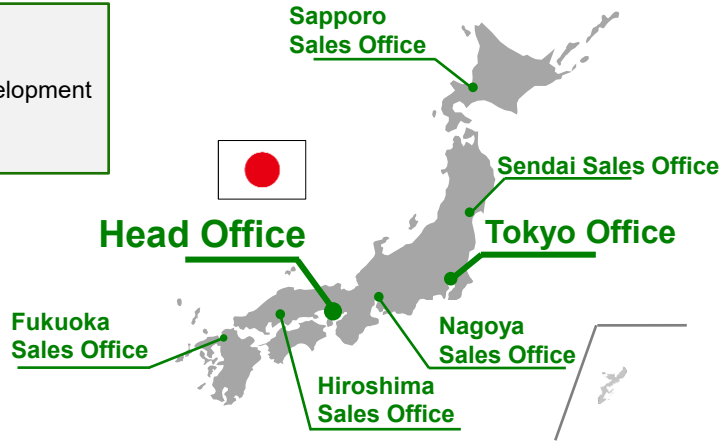

Seishin Plant

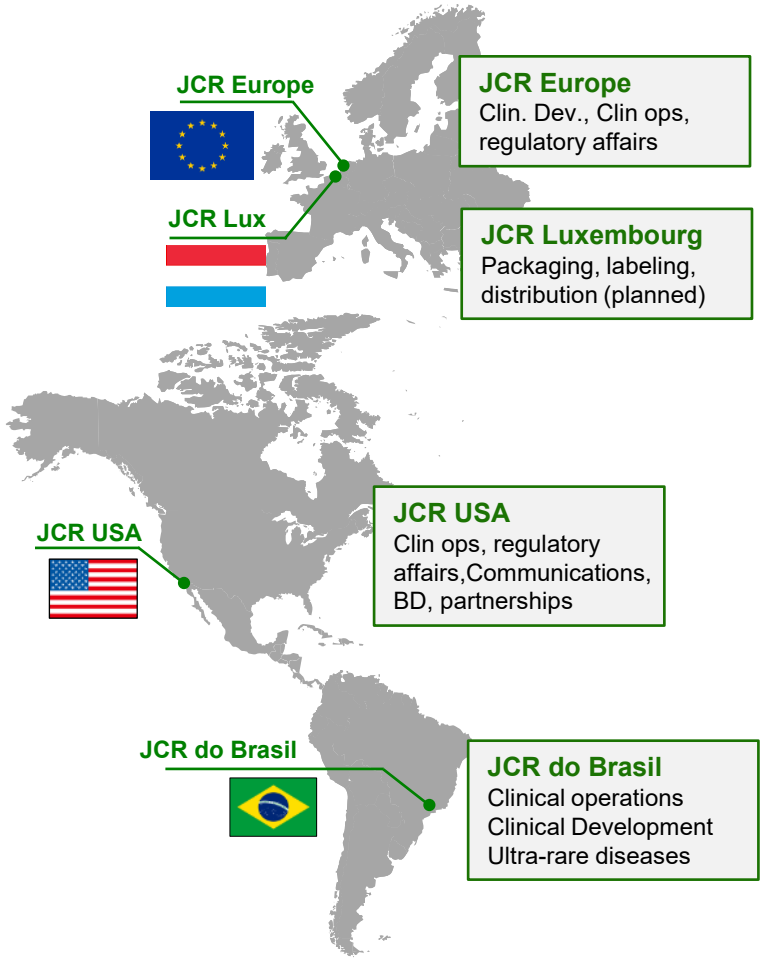

Kobe Plant


Research Institute


Kobe Science Park Ctr.

JCR Japan
Sales
Research & Development
Production
Corporate HQ





JCR Europe
Clin. Dev., Clin ops, regulatory affairs

JCR Luxembourg
Packaging, labeling, distribution (planned)

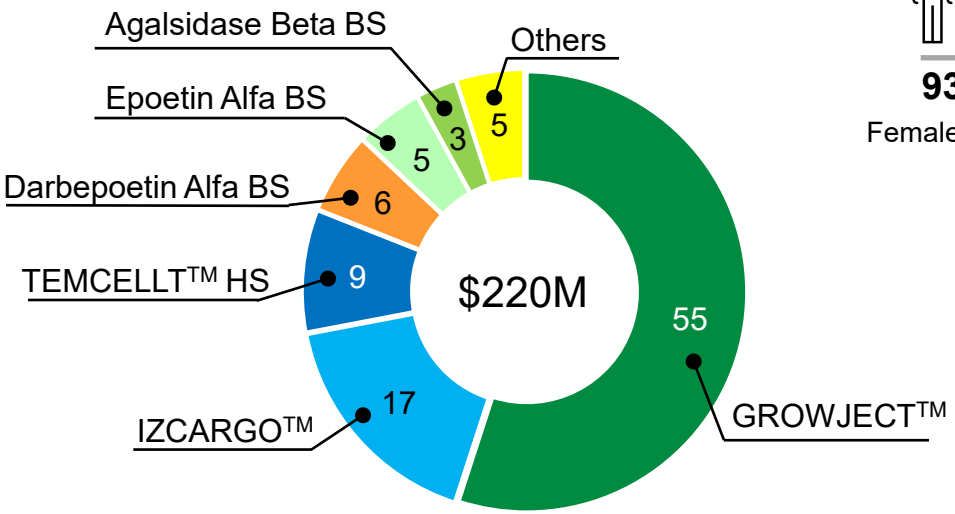
JCR USA
Clin ops, regulatory affairs,Communications, BD, partnerships

JCR do Brasil
Clinical operations
Clinical Development
Ultra-rare diseases

JCR is Fully Integrated Bio-Pharma Company from Research to Commercialization

Revenue Stream from a Diversified Domestic Portfolio

Percentage of Total Sales (FY2024)

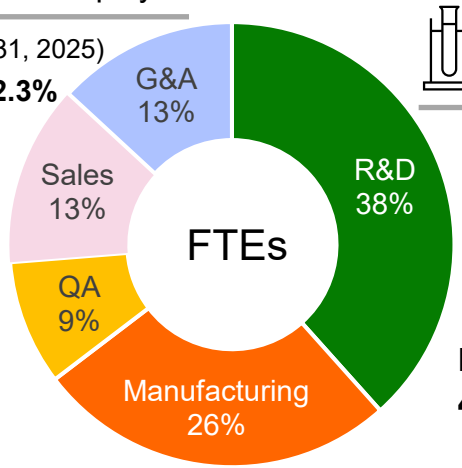


Allocation of FTEs (FY2024)

Japan Employees

938 (Mar. 31, 2025)

Female ratio: **42.3%**



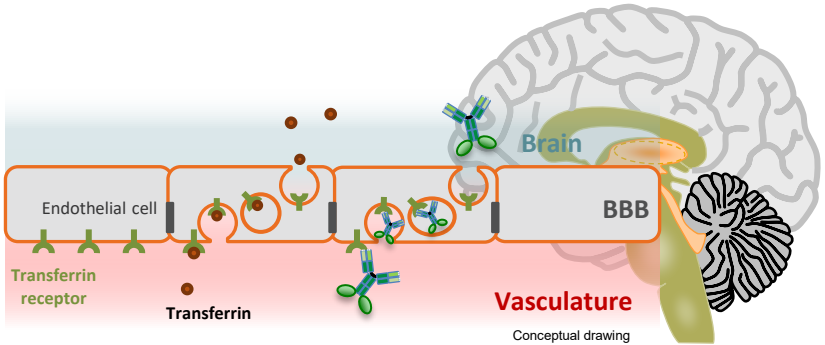
R&D Pipeline

17+ LSD assets
1 growth hormone
1 DMD program

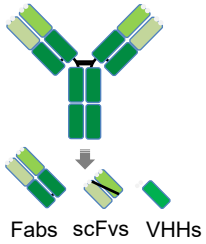
R&D Expense Ratio:
46.7% (FY2024)

JCR Developed the World's First Commercialized Brain Shuttle Medicine

2021
Pabinafusp alfa (IZCARGO™) was approved in Japan for the treatment of MPS II.



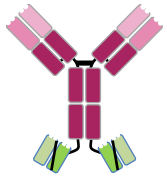
Platform expansion into new indications

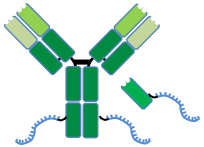


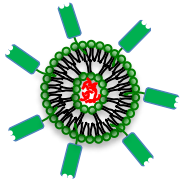
2026

Neuro Degeneration	Neuro inflammation
Neuro Oncology	Neuro-muscular Diseases

Therapeutic mAb delivery to the CNS	Gene therapy delivery to the CNS
ASO delivery to the CNS or muscle	LNPs: CNS delivery of mRNA or siRNA







LSD: Lysosomal storage disease TfR: Transferrin receptor BBB: Blood Brain Barrier CNS: Central nervous system

An Industry-Leading LSD Portfolio based on the J-Brain Cargo Technology

Portfolio (preclinical and clinical)

To all patients around the world

Marketed

- JR-141 MPS II (Hunter) — Approved (JP)
- Fabry*

Clinical

- JR-141 MPS II (Hunter) — Phase III (global)
- JR-171 MPS I (Hurler, etc.)
- JR-441 MPS IIIA (Sanfilippo A)
- JR-446 MPS IIIB (Sanfilippo B) — Phase I/II

Preclinical

- JR-162 Pompe
- JR-479 GM2 gangliosidosis
- JR-471 Fucosidosis
- JR-443 MPS VII (Sly)
- GT CLN1 (Batten type I)
- GT CLN2 (Batten type II)
- GM1 gangliosidosis
- Niemann-Pick
- MLD
- Galactosialidosis
- α-Mannosidosis
- Gaucher
- Krabbe

Research phase

Partnered with Medipal

JCR Pharmaceuticals × MEDIPAL

JR-471 JR-446 JR-479

● Protein therapy ● Gene therapy

* Agalsidase beta biosimilar (not based on JBC platform)

Overview of Clinical or Late Preclinical Pipeline

Code	Indication	Status	Milestones/Comments
		Preclinical / Phase 1 / Phase 2 / Phase 3	
JR-141	MPS II (Hunter syndrome)	Global Ph3	• On track for ~FY2027 approval in US, EU, Brazil.
JR-142	Pediatric GHD	Ph3 (Japan)	• Patient recruitment on track.
JR-171	MPS I (Hurler syndrome etc.)	Global Ph1/2 completed	• Three-year phase 1/2 study completed. • Partnering activities ongoing.
JR-441	MPS IIIA (Sanfilippo syndrome type A)	Ph1/2 (Germany) Ph1 (Japan)	• Ph1/2 1-year data for the initially planned dose groups. • Actively pursuing early approval in Japan.
JR-446	MPS IIIB (Sanfilippo syndrome type B)	Ph1/2 (Japan)	• Promising and consistent efficacy with good safety profile. • Actively pursuing early approval in Japan • Partnered with MEDIPAL HOLDINGS
JR-471	Fucosidosis		• Commencement of natural history study • Partnered with MEDIPAL HOLDINGS
JR-479	GM2 gangliosidosis (Tay-Sachs disease, Sandhoff disease)		• Partnered with MEDIPAL HOLDINGS
Givinostat	Duchenne muscular dystrophy	Approved in key markets worldwide	• Under discussions with PMDA regarding early submission pathway.

Partnerships are at the Core of JCR's Business Philosophy

Approval of IZCARGO™

JR-141 partnership with Takeda (until 2024)

Gene therapy partnership with Takeda (until 2024)

Ultra rare disease partnership with MEDIPAL

JBC partnership with Alexion (Oligonucleotides)

JBC partnership with Alexion (Neuro)

JBC partnership with Angelini (epilepsy)

Gene therapy capsid partnership with Alexion

Neurodegeneration partnership with Acumen

Givinostat partnership (Japan) Strategic partnership

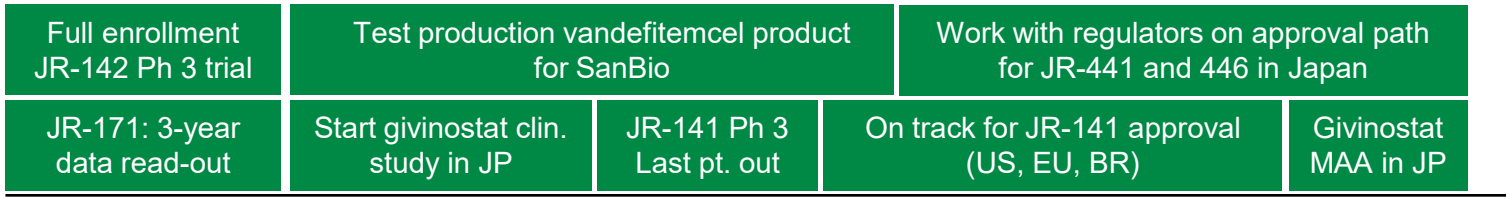
2021 2022 2023 2025

JCR's Growth Strategy

Key initiatives for sustained company growth

1. Advance internal portfolio in LSDs and GH deficiency (JR-141, JR-441, JR-446, JR-142, others)
2. Expand international footprint through platform, portfolio and strategic partnerships (e.g. Italfarmaco)
3. Strengthen domestic portfolio (e.g. givinostat)
4. Advance JUST-AAV platform towards clinical PoC (e.g. Alexion partnership)
5. Revenue stream through manufacturing of complex regenerative medicine products (e.g. vandefitemcel)

Prospective key events over the next 18-24 months (approximate timeline only)



A Strategic Partnership between JCR and Italfarmaco

Family-owned company
EUUS focus; presence in 90 countries
Proprietary mall molecule product portfolio

Strong Founder engagement
Japan footprint
Biologics product portfolio
Multiple Biotherapeutics platforms

Potential future expansion of the partnership

- JCR to enable Italfarmaco to enter into Biotherapeutics
- JCR to serve as preferred commercialization partner in Japan
- Italfarmaco to serve as preferential commercialization partner ex Japan

Address other muscular diseases using JCR platform technologies including genetic medicine

Joint development of other Biologics	Cross-licensing of other products	Preferential access to other (gene-) therapies / Biologics

Strategic partnership on Biotherapeutics

Givinostat Partnership with Italfarmaco

Since its first approval in 2024, Givinostat has been administered to thousands of individuals diagnosed with Duchenne's muscular dystrophy (DMD).

In December 2025, JCR has acquired the development and commercialization rights for givinostat for Japan.

Givinostat	Oral suspension, administered twice daily
Mode of action	HDAC inhibitor, reducing inflammation and fibrosis in muscle tissues; slows progression of DMD. Mutation agnostic MoA.
Approvals	Approved by the US, EU, UK, Israel and UAE regulatory authorities (under the trademark Duvyzat®); additional regulatory submissions are ongoing.
Value proposition	First oral non-steroid treatment for DMD, clinically shown to slow disease progression (4-step climb test), addressing high unmet need in DMD

Commercial Potential of Givinostat for the Treatment of DMD Patients in Japan

Duchenne muscular dystrophy is an indication with high unmet medical need and a significant patient population in Japan

- ~3,500 individuals in Japan diagnosed with DMD
- Over 1,000 subjects meet eligibility criteria per EMA prescription information (≥6 year of age; ambulatory and in transition[1]).
- Over 3,000 individuals with DMD are ≥6 years of age[1].
- Only two non-steroidal DMD treatments are approved in Japan[2].
- Premium prices are charged for existing non-steroid DMD therapies in Japan[3].

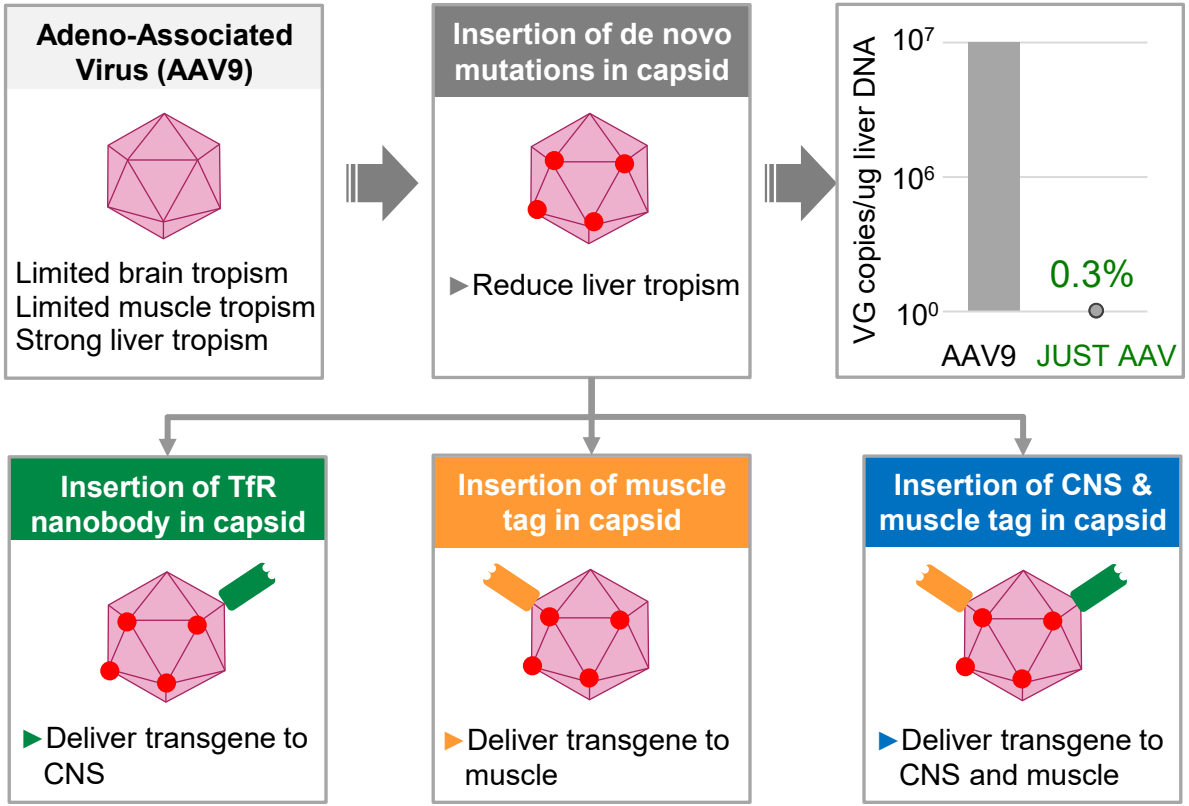
A substantial patient base and significant unmet need underpin strong sales potential in Japan

[1] Source: Remudy (Registry of Muscular Dystrophy)
[2] delandistrogene moxeparvovec-rokl (DMD patients age 3-7 year-old with no exon 8 or 9 deletions), viltolarsen (DMD patients amenable to exon 53 skipping therapies)
[3] Viltolarsen annual treatment costs (published base price are ~USD250,000 (25 kg bodyweight) – USD450,000 (45 kg bodyweight)

JUST-AAV: Unlocking New Frontiers in Gene Therapy

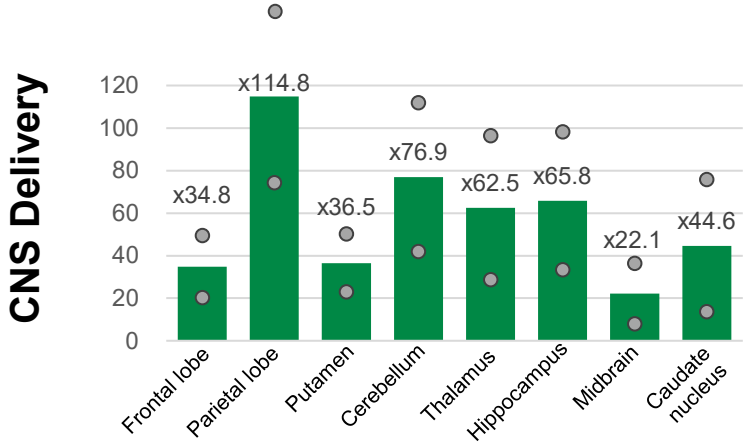
A novel AAV platform for targeted transgene delivery to the brain and other tissues and organs

Creation of "tags" with high tropism to other organs

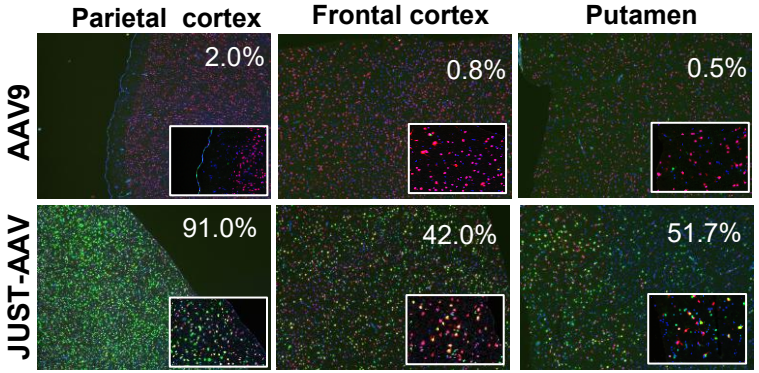


Engineering Directionality to Desired Tissues and Organs

Transgene mRNA expression (normalized to AAV9)

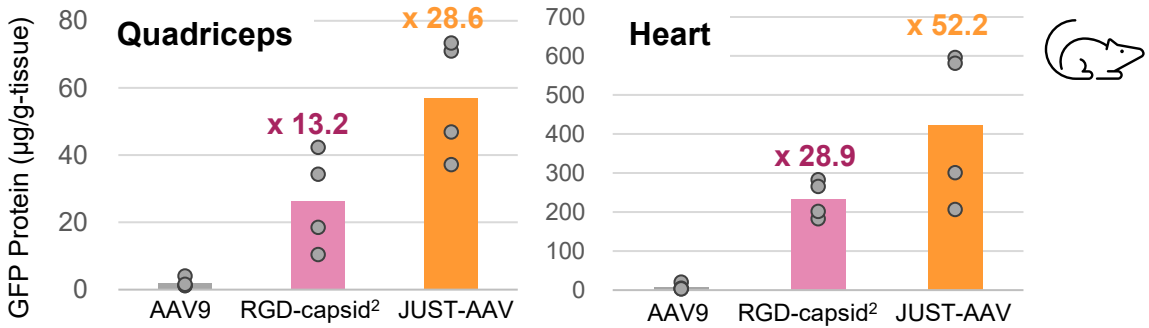


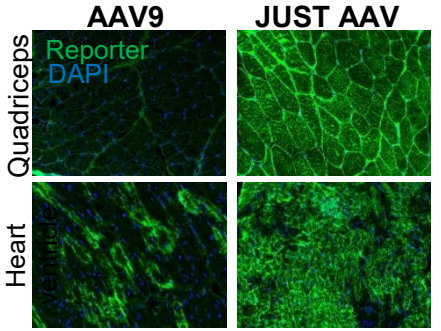
Quantitation of transduced cells[1]



Red = neuronal cells Green = expressed transgene (HA-H2B)

Muscle Delivery





[1] JCR internal data.

[2] Tabebordbar M, et al., *Cell*. 2021; 184(19): 4919-4938. e22.

The First International Partnership on the JUST-AAV Platform

July 2025

License agreement with Alexion for JUST-AAV capsids

- Alexion may use the licensed capsids, which are part of the JUST-AAV platform, in **up to five of Alexion's genomic medicine programs**

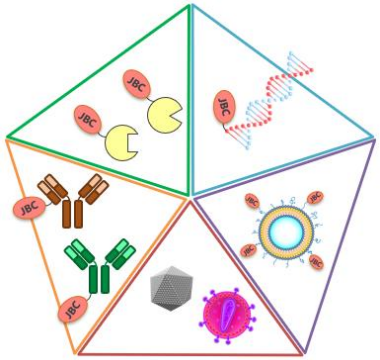
- **Milestone payments of up to USD 825 million**
  - Research and development : Up to USD 225 million
  - Commercial : Up to USD 600 million

The third partnership with Alexion, following research collaborations involving neurodegenerative disease and oligonucleotide therapeutics

Shaping the Future with JCR's Proprietary Technologies

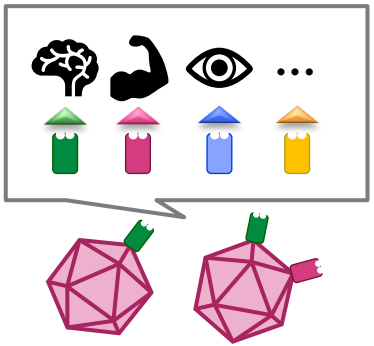
JBC and JUST-AAV enable multi-modal approaches and expand opportunities for out-licensing, driving long-term corporate value

J-Brain Cargo



Blood-Brain Barrier transport applicable to various modalities

JUST-AAV



AAV with enhanced delivery to target tissues and reduced liver tropism

- Neurodegeneration
- Muscular Diseases
- Rare Diseases
- Neuroinflammation
- Neuro-oncology
- ...

50th ANNIVERSARY

JCR Pharmaceuticals

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